
Stem Cells Secreting GDNF for the Treatment of ALS

Grant Award Details

Stem Cells Secreting GDNF for the Treatment of ALS

Grant Type: Disease Team Therapy Planning I

Grant Number: DR2-05320

Investigator:

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|---------------------|-----------------------------|
| Name: | Clive Svendsen |
| Institution: | Cedars-Sinai Medical Center |
| Type: | PI |

Disease Focus: Amyotrophic Lateral Sclerosis, Neurological Disorders

Award Value: \$63,487

Status: Closed

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Stem Cells Secreting GDNF for the Treatment of ALS

Public Abstract:

This project aims to use a powerful combined stem cell and gene therapy approach to treat patients with amyotrophic lateral sclerosis (ALS or Lou Gehrig's Disease). ALS is a devastating disease for which there is no treatment or cure. Progression from early muscle twitches to complete paralysis and death usually happens within 4 years. Every 90 minutes someone is diagnosed with ALS in the USA, and every 90 minutes someone dies from ALS. In California the death rate is one person every one and a half days.

Stem cells have been shown to produce support cells for dying motor neurons called astrocytes which may slow down disease progression. Furthermore, many studies have shown that growth factors such as glial cell line-derived growth factor (or GDNF) can protect motor neurons from damage in a number of different animal models including those for ALS. However, delivering GDNF to the spinal cord has been almost impossible as it does not cross from the blood to the brain tissue. The idea behind the current proposal is to modify stem cells to produce GDNF and then transplant these cells into patients. A number of advances in human stem cell biology along with new surgical approaches has allowed us to put together this disease team approach – a first in man study to deliver cells modified to release a powerful growth factor that are expected to slow down the death of motor neurons and paralysis in patients.

The focus of the proposal will be to perform essential preclinical studies in both small and large animals that will establish optimal doses and safe procedures for translating this stem cell and gene therapy into human patients. The Phase 1 clinical study will include 30 ALS patients from the state of California. This will be the first time this type of stem cell and gene therapy has been available to any ALS patients in the world.

Statement of Benefit to California:

ALS is a devastating disease, and also puts a large burden on state resources through the need of full time care givers and hospital equipment. It is estimated that the cost of caring for an ALS patient in the late stage of disease while on a respiration is \$200,00-300,000 per year. While primarily a humanitarian effort to avoid suffering, this project will also ease the cost of caring for ALS patients in California if ultimately successful. As the first trial in the world to combine stem cell and gene therapy it will make California a center of excellence for these types of studies. This in turn will attract scientists, clinicians, and companies interested in this area of medicine to the state of California thus increasing state revenue and state prestige in the rapidly growing field of Regenerative Medicine.

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